



MEMORANDUM

March 7, 2014

From: Gil Sambrano, Ph.D., Associate Director Review
To: Independent Citizens Oversight Committee (ICOC)
Subject: Consideration of RFA 13-01: Duane Roth Disease Team Therapy Development Awards III, Application DR3-07201

The ICOC considered the recommendations of the GWG for applications submitted in response to the Disease Team Therapy Development III RFA at its meeting held December 12, 2013. Consideration of application DR3-07201 by the ICOC was deferred due to the applicant filing an appeal request based on a conflict of interest allegation on the morning of December 12. The applicant alleged that the GWG review of the proposed project may have been “tainted” by the “perceived lack of objectivity” of one member of the GWG. There was no specific basis to support a financial, professional or personal conflict as defined in the GWG conflict of interest policy. Nevertheless, we examined what influence this reviewer may have had on the scoring and final recommendation of the proposal. We examined notes of the discussion taken by CIRM science officers at the review meeting, examined individual reviewer scores, and written critiques. We also presented the facts of this matter to the GWG review chair. Based on this examination, we found no evidence that the reviewer had any significant influence on the score or the recommendation. The reviewer was not an assigned reviewer and therefore did not contribute a written critique to the panel. Consistent with the recollection of the review chair and CIRM science officers in attendance, the discussion notes suggest that this reviewer did not provide any comment either in favor or against the proposal. The individual score given by the reviewer was very close to the mean score and thus did not contribute to the broad standard deviation. In summary, there is nothing specific or substantive to support a conflict of interest, including no evidence that the reviewer would gain financially, professionally, or personally through a negative review of this project. The appeal request was therefore denied.

The applicant also submitted a request for reconsideration based on material new information. Although the applicant provided some information that is new, it did not directly address the main concern of reviewers and therefore did not provide adequate grounds for reconsideration. The request was denied. Nevertheless, CIRM staff took an additional step of seeking the opinion of two new expert reviewers and the GWG chair to assess the proposal with the new information provided by the applicant. In general, the new expert reviewers raised many of the key concerns about the proposal as the original GWG reviewers and did not find the new information compelling. The experts felt that there were significant weaknesses in clinical trial design including a target population that

was too heterogeneous and as such would impair the ability to get useful data. The experts also felt that the preclinical data did not provide support for an effect on the proposed patient population as the preclinical model used was not representative of the condition existing in the targeted patients. Consistent with the GWG assessment, the additional expert reviewers advised that some evidence of efficacy (in addition to safety) from another trial currently evaluating this same product in a different subgroup of patients should be acquired to better inform the scope and design of trials with additional subgroups, such as that proposed in this application. CIRM staff believes that this new assessment, which considered the new information provided by the applicant, supports the original GWG recommendation to not fund the application.